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NICE ENABLES ACCESS TO FIRST TREATMENT THAT TARGETS THE UNDERLYING PATHOPHYSIOLOGY OF RARE DISEASE X-LINKED HYPOPHOSPHATAEMIA (XLH) IN ADULTS[1]

Kyowa Kirin is delighted by the decision made today by the National Institute for Health and Care Excellence (NICE) to recommend CRYSVITA® for the treatment of adults living with X-linked hypophosphataemia (XLH) in England, Wales and Northern Ireland

London, England. 21 June 2024 – Adults living with the rare disease X-linked hypophosphataemia (XLH) in England, Wales and Northern Ireland will now have access to the first available treatment to tackle the underlying cause of XLH following NICE's decision to recommend burosumab (marketed as Crysvita) to treat adults who have a confirmed diagnosis of XLH, following a new access deal.[1]

This decision ensures equal access to burosumab across the UK, as adults living with XLH in Scotland have been able to access burosumab on the NHS since March 2023, following approval by the Scottish Medicines Consortium (SMC) through their ultra-orphan pathway.[2] Burosumab is also available for eligible children and adolescents in all four UK nations.

Initially, NICE published an interim negative decision in its Appraisal Consultation Document (ACD). Following constructive engagement between Kyowa Kirin, NICE and NHS England, this recommendation was revised and an agreement for long term commissioning reached, thus resulting in a positive decision in its Final Draft Guidance (FDG).

XLH is a very rare genetic condition, affecting roughly 1.7 – 4.8 per 100,000 people.[3] This rare metabolic bone condition is a life-long and progressive disease that typically presents in early childhood, causing bowed legs, stunted growth, and bone and joint pain.[4] It is a whole life, whole body, and whole family disease, according to people living with the condition, as several family members are often impacted due to its inherited nature.[5] XLH also has an adverse impact on emotional wellbeing for various reasons including pain, uncertainty about the future, and may also be associated with financial challenges.[5,6] XLH does not just affect individuals with the disease, but also their family and friends, who are often involved in their support and care.[6]

Responding to NICE's final recommendation:

Oliver Gardiner, Trustee and Co-Founder of XLH UK, said: "This is fantastic news for adults living with XLH in England and Wales, for whom treatment options are currently severely limited and who, as a result of this recommendation, will have access to an effective treatment".

Dr Gavin Clunie, Consultant Rheumatologist and Metabolic Bone Physician at Addenbrooke's Hospital, said: "It is welcome that NICE have decided to recommend burosumab for the treatment of adults living with XLH, given the clinical benefit to these patients and significant unmet need. This is a milestone development for those adults living with XLH who need this treatment."

Emma Claeys, General Manager UK, Kyowa Kirin, said: "We are pleased that, following positive engagement with NICE and feedback from the patient and clinical community, this positive outcome demonstrates Kyowa Kirin's commitment to delivering access to innovative treatments to people living with rare diseases in the United Kingdom."

Burosumab is currently the only licensed medicine that addresses the underlying causes (pathophysiology) of XLH, rather than only the symptoms. The drug is now reimbursed in several European countries for both the paediatric and adult populations, including France, Germany, Italy and Spain.[7,8,9]

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Notes to editor:

About X-linked hypophosphataemia

XLH is caused by a genetic mutation which leads to overexpression of the protein FGF23, a protein involved in the regulation of phosphate concentration in the blood. In XLH, FGF23 is produced in excess leading to depletion of phosphate in the blood, known as hypophosphataemia.[10]

Individuals living with the disease may display a multitude of symptoms including short stature, limb deformities, bone and joint pain, oral abscesses, and hearing loss.[11] To manage this wide variety of symptoms, the disease is managed through multi-disciplinary teams.[12]

About CRYSVITA® (burosumab) in XLH

Burosumab is a recombinant human monoclonal antibody that binds to the protein FGF23. This has the impact of inhibiting the action of FGF23, allowing phosphate regulation in the body to be restored.[12]

In 2018, the European Commission granted a conditional marketing authorisation for burosumab for the treatment of XLH with radiographic evidence of bone disease in children one year of age and older and in adolescents with growing skeletons.[13] Following this, the European Commission granted burosumab a conditional marketing authorisation in 2020, for the treatment of adolescents regardless of growth status and adults with XLH.[14] The licence was then converted from a conditional to a full standard marketing authorisation in 2022,[15] and has been approved by the MHRA via the reliance route.[16] The drug is now reimbursed in several European countries for both the paediatric and adult population, including France, Germany and Italy.[7,8,9]

About Kyowa Kirin

Kyowa Kirin strives to create and deliver novel medicines with life-changing value. As a Japan based global specialty pharmaceutical company with a heritage of more than 70 years, the company applies cutting-edge science, including expertise in antibody research and engineering, to address the needs of patients across multiple therapeutic areas such as nephrology, oncology, immunology/allergy and neurology. Across its four regions – Japan, Asia Pacific, North America and EMEA/International – Kyowa Kirin focuses on its purpose, to make people smile, and is united by its shared values of commitment to life, teamwork, innovation and integrity.

You can learn more about Kyowa Kirin International at: https://international.kyowa-kirin.com

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